Correlation between MG Symptoms PRO and existing MG-specific outcome scores in the Phase 3 MycarinG study: *Post hoc* analysis

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Introduction

- The MG Symptoms PRO is a novel PRO assessment in MG, comprising five independent scales: Bulbar Muscle Weakness, Ocular Muscle Weakness, Respiratory Muscle Weakness, Muscle Weakness Fatigability and Physical Fatigue (**Table 1**) [1]
  - The independent scales were developed to capture patients' perspectives on MG symptoms over time and were used to assess treatment efficacy in the Phase 3 MycarinG (NCT03971422) study[1,2]
- The double-blind, placebo-controlled MycarinG study evaluated the efficacy and safety of rozanolixizumab, a humanized IgG4 monoclonal antibody FcRn inhibitor, versus placebo in patients with gMG[2]
  - Clinically relevant and statistically significant reductions from baseline in MG-ADL (primary endpoint) and QMG (secondary endpoint) total scores were observed at Day 43 for both rozanolixizumab dose groups versus placebo (**Figure 1a and 1b**)[2]
  - Statistically significant reductions from baseline at Day 43 were also observed in MG Symptoms PRO Muscle Weakness Fatigability, Physical Fatigue and Bulbar Muscle Weakness scores versus placebo (secondary endpoints; **Figure 1c–e**)[2]
- This *post hoc* analysis aimed to evaluate the correlation between MG Symptoms PRO scale scores and MG-ADL and QMG subdomain scores using data from the MycarinG study

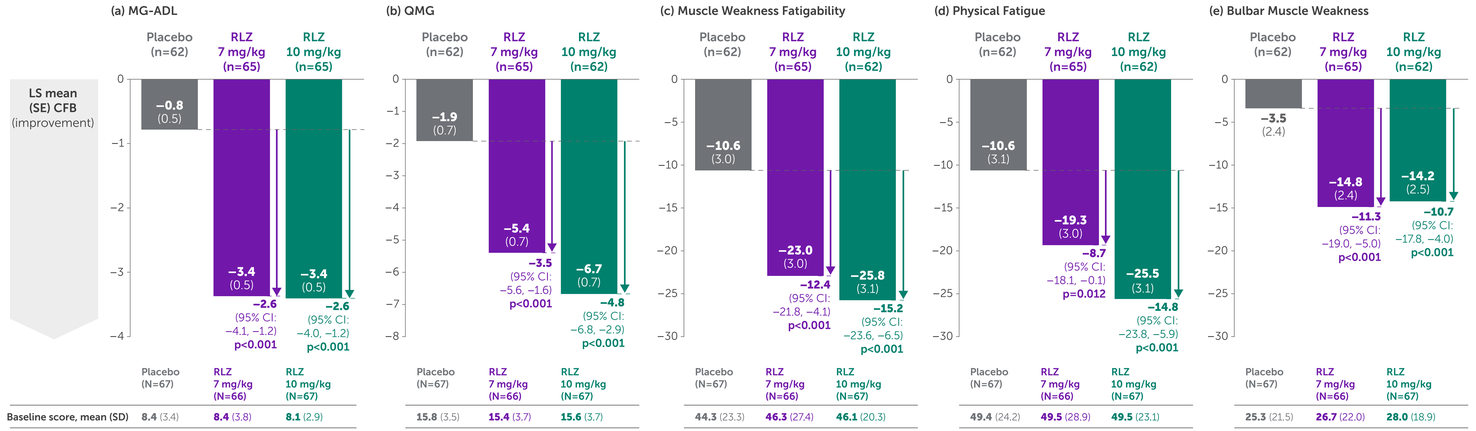
Methods

- Adults with MGFA Disease Class II–IVa anti-AChR Ab+ or anti-MuSK Ab+ gMG with an MG-ADL score ≥3 (for non-ocular symptoms) and a QMG score ≥11 were enrolled
- Correlations between the five MG Symptoms PRO scale scores and MG-ADL and QMG subdomain scores were evaluated *post hoc* using the Pearson coefficient to assess data from baseline, prior to rozanolixizumab administration
- Thresholds were applied to indicate the strength of correlations:
  - Weak: 0.3 to <0.5
  - Moderate: 0.5 to <0.7
  - Strong: ≥0.7

Results

- Overall, 200 patients entered the MycarinG study
- Baseline correlation coefficients between the MG Symptoms PRO scale scores and MG-ADL subdomain scores were strong (≥0.7) for domain-matched ocular and bulbar scores (**Figure 2**)
  - Correlation coefficients among MG Symptoms PRO scale scores and MG-ADL subdomain scores were lower among concepts not explicitly captured by the MG-ADL measure, namely Physical Fatigue and Muscle Weakness Fatigability
- Correlations between the MG Symptoms PRO scale scores and QMG subdomain scores were generally weak (<0.5; **Figure 3**)
  - A moderate correlation was observed between the Bulbar Muscle Weakness score and QMG bulbar subdomain score (0.50)
- Overall, TEAEs occurred in 81.3% (n=52/64), 82.6% (n=57/69) and 67.2% (n=45/67) of patients treated with rozanolixizumab 7 mg/kg, 10 mg/kg and placebo, respectively; most were mild/moderate

Figure 1 Improvements from baseline in (a) MG-ADL, (b) QMG and MG Symptoms PRO (c) Muscle Weakness Fatigability, (d) Physical Fatigue and (e) Bulbar Muscle Weakness scores were observed at Day 43 for both rozanolixizumab dose groups versus placebo[2]



Randomized set (all patients who were randomized, using the treatment assigned). Clinically meaningful response was defined as a ≥2.0-point or ≥3.0-point improvement from baseline in MG-ADL and QMG score, respectively.[3,4]

Summary and conclusions

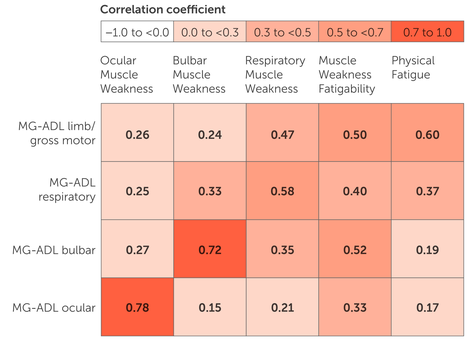
This *post hoc* analysis evaluated the correlation between MG Symptoms PRO scale scores and MG-ADL and QMG subdomain scores using baseline data from the Phase 3 MycarinG study

Moderate and strong correlations were observed between all five MG Symptoms PRO scale scores and the relevant MG-ADL subdomain scores, reflecting similar concepts between the two measures

As expected, weaker correlations were observed between MG Symptoms PRO scale scores and QMG subdomain scores, likely due to the different perspectives captured through patient-reported versus clinician-assessed data, and different recall times

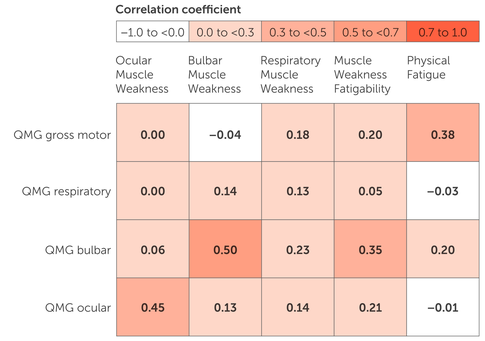
The MG Symptoms PRO scales provide a granular assessment of the broad-ranging symptoms of MG, complementing existing outcome measures by capturing important patient-relevant experiences, such as physical fatigue, that are not assessed by other MG-specific outcome measures

Figure 2 Moderate and strong correlations were observed between all five MG Symptoms PRO scale scores and relevant MG-ADL subdomain scores



Randomized set.

Figure 3 Correlations between MG Symptoms PRO scale scores and QMG subdomain scores were generally weak



Randomized set.

Table 1 Overview of measures

	Ocular Muscle Weakness/ocular	Bulbar Muscle Weakness/bulbar	Respiratory Muscle Weakness/respiratory	Limb weakness/ gross motor	Muscle Weakness Fatigability	Physical Fatigue
MG-ADL[5]	• Double vision • Eyelid droop	• Talking • Swallowing • Chewing	• Breathing	• Impairment of ability to brush teeth or comb hair • Impairment of ability to arise from a chair		
QMG[3]	• Double vision on lateral gaze (right or left) • Ptosis (upward gaze) • Facial muscles	• Speech after counting aloud from 1 to 50 (onset of dysarthria) • Swallowing 4oz water	• Forced vital capacity	• Arms outstretched (2) • Legs outstretched (2) • Hand grip strength (2) • Head lifted		
MG Symptoms PRO	• Double vision • Eyelid drooping • Blurry vision • Difficulty moving eyes (2)	• Speech (2) • Swallowing (2) • Chewing • Difficulty controlling liquids in mouth • Mouth drooping • Voice (3)	• Breathing		Worsening over time of: • Leg and arm weakness (2) • Breathing • Talking (2) • Vision • Eyelid drooping • Chewing • Swallowing	• Physical tiredness (4) • Muscle weakness (4) and heaviness (3) • Physical difficulty in moving (4)

The value in brackets indicates the number of items assessed. In the MycarinG study, the recall period for MG-ADL was "current situation", the QMG (performance outcome) was used to assess current muscle strength and fatigability, and the recall period for the MG Symptoms PRO scales was the past 7 days.

Abbreviations: Ab+, antibody positive; AChR, acetylcholine receptor; CFB, change from baseline; CI, confidence interval; FcRn, neonatal Fc receptor; gMG, generalized myasthenia gravis; IgG4, immunoglobulin G4; LS, least squares; MG, myasthenia gravis; MG-ADL, Myasthenia Gravis Activities of Daily Living; MGFA, Myasthenia Gravis Foundation of America; MuSK, muscle-specific tyrosine kinase; PRO, patient-reported outcome; QMG, Quantitative Myasthenia Gravis; RLZ, rozanolixizumab; SD, standard deviation; SE, standard error; TEAE, treatment-emergent adverse event.

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